elevation of ALC (day 16) along with decrease in chimerism to 85%. This time alemtuzumab was used promptly, leading to successful outcome (Table 1).

Conclusion: In patients with Fanconi Anemia undergoing transplant, the development of mixed chimerism may be due to incomplete lympho-depletion and return of recipient lymphocytes. In these cases, prompt and aggressive lymphoid depleting therapy is warranted to prevent graft rejection.

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Hematopoietic Cell Transplant for Patients With NEMO Defect: A Single Center Experience

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Background: NF-kappa B essential modulator (NEMO) is an important activator of NF-kappa B, which regulates numerous genes involved in immune responses and ectodermal development. Hypomorphic mutations in NEMO have been associated with a spectrum of disorder such as X linked anhidrotic ectodermal dysplasia with immunodeficiency (XL-EDA-ID), X- liked immunodeficiency without ectodermal dysplasia and isolated susceptibility to atypical mycobacteria. Currently, allogeneic hematopoietic cell transplantation (HCT) is the only curative therapy for patients with NEMO defects. However, there are only few case reports of successful HCT for this disorder. Here we report our experience of allogeneic HCT for patients with NEMO defect.

Methods: We retrospectively analyzed the outcomes of 4 patients with NEMO defect who had undergone allogeneic HCT between January 2005 and September 2012 in our institution. Hypomorphic mutations in NEMO were identified in all 4 patients. Three had XL-EDA-ID and one had X-liked immunodeficiency without ectodermal dysplasia. Prior to transplant all of them had recurrent bacterial infections and two of them had disseminated atypical mycobacterial infection. Autoimmune hemolytic anemia was noted in two patients. The median age at transplantation was 10.5 years (range: 3.3-18.2). All of them underwent myeloablative preparative regimen consisting of busulfan, cyclophosphamide, and ATG, and busulfan kinetics were used to dose busulfan. Two patients received matched (8/8) unrelated donor (URD) bone marrow transplant and two received single locus mismatch (7/8) URD bone marrow transplant. In all patients cyclosporine and prednisone was used as GvHD prophylaxis.

Result: Overall survival was 75% (3/4) at a median follow-up of 59 months post-transplant (range: 12 -70). The median time for neutrophil engraftment was 12.5 days (range: 11-15). Grade II to III acute GVHD developed in two patients and one patient developed chronic skin GVHD. Mixed donor chimerism was noted in one patient needing stem cell boost 4 years after HCT. Death in one patient was due to HSV pneumonitis and disseminated mycobacterial infection with multi-organ failure. All surviving patients immune reconstituted with normal lymphocyte subpopulation, normal distribution of CD45RA/RO, normal NK cell function and mitogen response. Two patients are currently off immunoglobulin replacement with good specific antibody response to both protein and polysaccharide antigens.

Conclusion: HCT is effective in correction of innate and adaptive immune defects associated with NEMO deficiency. Further follow-up and larger multi-institutional studies are needed to better understand the role of HCT in this disorder.

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Autologous Hematopoietic Stem Cell Transplantation in a Single Pediatric Center

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After the first five years working we evaluate the results of Autologous Stem Cell Transplantation (ASCT) performed between 2007 and 2012, a total of 25 ASCT, 16 neuroblastomas, 6 Hodgkin Diseases, 1 LBCL, 1 AML, 1 PNET. Median age was 7.2 years, 13 females, 12 males. Conditioning regimens were 17 CEM, 5 BU/MEL, 3 BEAM. Stem cell source was 21 PBSC, 2 PBSC + BM, 2 BM. The median MNC and CD34 infused were 5.3 x 10^8/kg and 4.21 x 10^6/kg. Hematological recovery median time was 11 days for neutrophils 12 days for platelets. Hospitalization median time was 34 days, two patients (SDRA- graft syndrome and gastrointestinal bleeding) required transfer to the intensive care unit, both of them improved and discharged. One hundred days mortality was 12% (3 patients) all for relapse of the underlying disease. Of a total of 25 patients, 9 patients (36%) have died, all cases because of disease relapse. These data show a very low transplant related mortality as expected for ASCT, and demonstrate that autologous transplantation in pediatric patients can be safely and effectively performed in developing countries with results comparable to those obtained in developed countries. Our goal in the future is to assess the long term effects on survivors and show similar results in allogeneic related and unrelated transplants currently under evaluation.

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Role of Conditioning Regimens in Pediatric Hematopoietic Stem Cell Transplant for Malignant Disorders: Reduced Toxicity Versus Standard Myeloablative Regimens

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Introduction: Allogeneic hematopoietic stem cell transplantation (HSCT) for malignant disorders is complicated by the risk of early and late treatment related toxicities. Reduced toxicity conditioning (RTC) regimens using Busulfan/Fludarabine (Bu/Flu) +/- 400Gy of Total Body Irradiation (TBI) were designed to lower the risk of these complications while achieving similar outcomes to the standard myeloablative conditioning (sMAC) with Busulfan/Cyclophosphamide (Bu/Cy) or 1200 Gy TBI/ Cyclophosphamide (TBI/Cy). Methods We compared outcomes of children conditioned with sMAC versus RTC pre-HSCT between Jan 2008 and Dec 2011. In both regimens, the total dose of Bu was adjusted to target an AUC of 16000 μM*min. Long term outcome analysis included assessment of change in pulmonary function test (PFT), cardiac function, thyroid function and growth from baseline. Patients with GVHD of the lung were excluded for assessing pulmonary function. Results Sixty seven patients with AML/MDS (n=29), ALL (n= 32),